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## California's Stem Cell Agency Supports Orchard Therapeutics' Acquisition of GSK Gene Therapy Programs for Rare Diseases

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**Oakland, CA** – The recent agreement transferring GSK's rare disease gene therapies to Orchard Therapeutics was good news for both companies and for the patients who are hoping this research could lead to new treatments, even cures, for some rare diseases. It was also good news for the California Institute for Regenerative Medicine (CIRM), the state's stem cell agency, which played a key role in helping Orchard grow to the point where this deal was possible.

At CIRM, our value proposition is centered around our ability to advance the field of regenerative medicine in many different ways," says Maria T. Millan, President & CEO of CIRM. "CIRM's funding and partnership has enabled the smooth transfer of Dr. Kohn's technology from the academic to the industry setting while conducting this important pivotal clinical trial. With our help, Orchard was able to attract more outside investment and now it is able to grow its pipeline utilizing this platform gene therapy approach."

Under the deal, GSK not only transfers its rare disease gene therapy portfolio to Orchard, it also becomes a shareholder in the company with a 19.9 percent equity stake. GSK is also eligible to receive royalties and commercial milestone payments. This agreement is both a recognition of Orchard's expertise in this area, and the financial potential of developing treatments for rare conditions.

For CIRM it's further proof that the agency's impact on the field of regenerative medicine extends far beyond the funding it offers companies like Orchard.

"Accelerating stem cell therapies to patients with unmet medical needs involves a lot more than just funding research; it involves supporting the research at every stage and creating partnerships to help it fulfill its potential," says Dr. Millan. "We invest when others are not ready to take a chance on a promising but early stage project. That early support not only helps the scientists get the data they need to show their work has potential, but it also takes some of the risk out of investments by venture capitalists or larger pharmaceutical companies."

CIRM's early support helped UCLA's Don Kohn, MD, develop a stem cell therapy for severe combined immunodeficiency (SCID). This therapy is now Orchard's lead program in ADA-SCID, OTL-101. OTL-101 has transformed the lives of dozens of children born with this usually fatal immune disorder.

"This gene correction approach for severe combined immunodeficiency (SCID) has already transformed the lives of dozens of children treated in early trials and CIRM is pleased to be a partner on the confirmatory trial for this transformative treatment for patients born with this fatal immune disorder," says Sohel Talib, CIRM's Associate Director Therapeutics and Industry Alliance.

Dr. Kohn, now a member of Orchard's scientific advisory board, said: "CIRM funding has been essential to the overall success of my work, supporting me in navigating the complex regulatory steps of drug development, including interactions with FDA and toxicology studies that enhanced and helped drive the ADA-SCID clinical trial."

CIRM funding has allowed Orchard Therapeutics to expand its technical operations footprint in California, which now includes facilities in Foster City and Menlo Park, bringing new jobs and generating taxes for the state and local community.

Mark Rothera, Orchard's President and CEO, commented: "The partnership with CIRM has been an important catalyst in the continued growth of Orchard Therapeutics as a leading company transforming the lives of patients with rare diseases through innovative gene therapies. The funding and advice from CIRM allowed Orchard to accelerate the development of OTL-101 and to build a manufacturing platform to support our development pipeline which includes 5 clinical and additional preclinical programs for potentially transformative gene therapies".

Since CIRM was created by the voters of California the Agency has been able to use its support for research to leverage an additional \$1.9 billion in funds for California. That money comes in the form of co-funding from companies to support their own projects,

partnerships between outside investors or industry groups with CIRM-funded companies to help advance research, and additional funding that companies are able to attract to a project because of CIRM funding.

#### **About CIRM**

At CIRM, we never forget that we were created by the people of California to accelerate stem cell treatments to patients with unmet medical needs, and act with a sense of urgency to succeed in that mission.

To meet this challenge, our team of highly trained and experienced professionals actively partners with both academia and industry in a hands-on, entrepreneurial environment to fast track the development of today's most promising stem cell technologies.

With \$3 billion in funding and approximately 300 active stem cell programs in our portfolio, CIRM is the world's largest institution dedicated to helping people by bringing the future of cellular medicine closer to reality.

For more information go to [www.cirm.ca.gov](http://www.cirm.ca.gov)

#### **About Orchard**

Orchard Therapeutics is a leading global fully integrated commercial stage company dedicated to transforming the lives of patients with rare diseases through innovative gene therapies.

Orchard's portfolio of autologous *ex vivo* gene therapy programs has demonstrated sustained clinical benefit in over 120 patients across five disease areas. These programs include Strimvelis, the first autologous *ex vivo* gene therapy approved by the EMA in 2016, 3 programs in advanced registrational studies in MLD (metachromatic leukodystrophy), WAS (Wiskott Aldrich syndrome) and ADA-SCID (adenosine deaminase severe combined immunodeficiency), other clinical programs in X-CGD (X-linked chronic granulomatous disease) and beta-thalassemia, as well as an extensive preclinical pipeline.

The company is partnered with world-leading institutions in gene therapy, including University College London, Great Ormond Street Hospital, the University of Manchester and Central Manchester University Hospitals, the University of California Los Angeles, Boston Children's Hospital, and Telethon Institute of Gene Therapy/Ospedale San Raffaele.

Orchard is privately held with offices in the UK and the US, including London, San Francisco and Boston. The company raised \$110 million in a Series B in December 2017, was named a Fierce 15 Company by FierceBiotech in 2016, and was awarded a \$19 million grant from the California Institute of Regenerative Medicine (CIRM).

For further information please visit [www.orchard-tx.com](http://www.orchard-tx.com)

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